

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



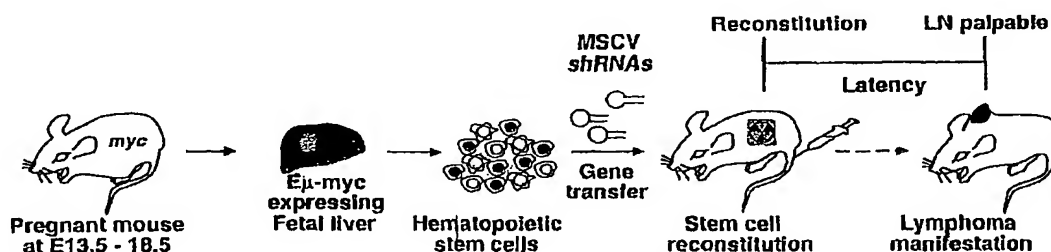
(43) International Publication Date
8 April 2004 (08.04.2004)

PCT

(10) International Publication Number
WO 2004/029219 A2

- (51) International Patent Classification⁷: **C12N**
- (21) International Application Number:
PCT/US2003/030901
- (22) International Filing Date:
29 September 2003 (29.09.2003)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data:
60/414,605 27 September 2002 (27.09.2002) US
- (71) Applicant (for all designated States except US): **COLD SPRING HARBOR LABORATORY** [US/US]; One Bungtown Road, Cold Spring Harbor, NY 11724 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): **FRIDMAN, Jordan** [US/US]; 4 East Mill Drive, Apt. 1A, Great Neck, NY 11021 (US). **HANNON, Gregory, J.** [US/US]; 92 Sammis Street, Huntington, NY 11743 (US). **HEMANN, Michael** [US/US]; 151 Lexington Avenue, Oyster Bay, NY 11771 (US). **LOWE, Scott, W.** [US/CA]; One Bungtown Road, Cold Spring Harbor, NY 11724 (US). **PADDISON, Patrick, J.** [US/US]; 46 Bayside Avenue, Oyster Bay, NY 11771 (US). **ZILFOU, Jack** [US/US]; 2233 Liberty Street, 2nd Floor, Allentown, PA 18104 (US). **DICKINS,**
- (84) Designated States (regional): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- (74) Agents: **QUISEL, John, D.** et al.; Ropes & Gray LLP, One International Place, Boston, MA 02110-2624 (US).
- (81) Designated States (national): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- Published:**
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: CELL-BASED RNA INTERFERENCE AND RELATED METHODS AND COMPOSITIONS



(57) Abstract: The invention provides, among other things, methods for performing RNA interference in stem cells and methods for using the stem cells in vivo.